

seq id.ST25

SEQUENCE LISTING

<110> Mirus Corporation  
 Wong, So  
 Wakefield, Darren  
 Sokoloff, Alex  
 Monahan, Sean  
 Sebestyen, Magdolna  
 Wolff, Jon  
 Higgs, Lori

<120> A COMPOUND FOR TARGETING TO HEPATOCYTES IN VIVO

<130> Mirus.014.06

<160> 36

<170> PatentIn version 3.1

<210> 1

<211> 33

<212> PRT

<213> Bacteriophage T7

<400> 1

Lys Asn Glu Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp  
 1 5 10 15

Glu Thr Lys Gly Phe Arg Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala  
 20 25 30

Gly

<210> 2

<211> 33

<212> DNA

<213> Bacteriophage T7

<400> 2

aaggagggtca tatggctaac gtaattaaaa ccg 33

<210> 3

<211> 39

<212> DNA

<213> Bacteriophage T7

<400> 3

gattggatcc ttactcgttc tccaccatga ttgcattag 39

<210> 4

<211> 573

<212> PRT

<213> Bacteriophage T7

<400> 4

seq id.ST25

Met Gly Ser Ser His His His His His His Ser Ser Gly Leu Val Pro  
1 5 10 15

Arg Gly Ser His Met Ala Asn Val Ile Lys Thr Val Leu Thr Tyr Gln  
20 25 30

Leu Asp Gly Ser Asn Arg Asp Phe Asn Ile Pro Phe Glu Tyr Leu Ala  
35 40 45

Arg Lys Phe Val Val Val Thr Leu Ile Gly Val Asp Arg Lys Val Leu  
50 55 60

Thr Ile Asn Thr Asp Tyr Arg Phe Ala Thr Arg Thr Thr Ile Ser Leu  
65 70 75 80

Thr Lys Ala Trp Gly Pro Ala Asp Gly Tyr Thr Thr Ile Glu Leu Arg  
85 90 95

Arg Val Thr Ser Thr Thr Asp Arg Leu Val Asp Phe Thr Asp Gly Ser  
100 105 110

Ile Leu Arg Ala Tyr Asp Leu Asn Val Ala Gln Ile Gln Thr Met His  
115 120 125

Val Ala Glu Glu Ala Arg Asp Leu Thr Thr Asp Thr Ile Gly Val Asn  
130 135 140

Asn Asp Gly His Leu Asp Ala Arg Gly Arg Arg Ile Val Asn Leu Ala  
145 150 155 160

Asn Ala Val Asp Asp Arg Asp Ala Val Pro Phe Gly Gln Leu Lys Thr  
165 170 175

Met Asn Gln Asn Ser Trp Gln Ala Arg Asn Glu Ala Leu Gln Phe Arg  
180 185 190

Asn Glu Ala Glu Thr Phe Arg Asn Gln Ala Glu Gly Phe Lys Asn Glu  
195 200 205

Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp Glu Thr Lys  
210 215 220

Gly Phe Arg Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala Gly Gln Tyr  
225 230 235 240

Ala Thr Ser Ala Gly Asn Ser Ala Ser Ala Ala His Gln Ser Glu Val  
245 250 255

seq id.ST25

Asn Ala Glu Asn Ser Ala Thr Ala Ser Ala Asn Ser Ala His Leu Ala  
 260 265 270  
 Glu Gln Gln Ala Asp Arg Ala Glu Arg Glu Ala Asp Lys Leu Glu Asn  
 275 280 285  
 Tyr Asn Gly Leu Ala Gly Ala Ile Asp Lys Val Asp Gly Thr Asn Val  
 290 295 300  
 Tyr Trp Lys Gly Asn Ile His Ala Asn Gly Arg Leu Tyr Met Thr Thr  
 305 310 315 320  
 Asn Gly Phe Asp Cys Gly Gln Tyr Gln Gln Phe Phe Gly Gly Val Thr  
 325 330 335  
 Asn Arg Tyr Ser Val Met Glu Trp Gly Asp Glu Asn Gly Trp Leu Met  
 340 345 350  
 Tyr Val Gln Arg Arg Glu Trp Thr Thr Ala Ile Gly Gly Asn Ile Gln  
 355 360 365  
 Leu Val Val Asn Gly Gln Ile Ile Thr Gln Gly Gly Ala Met Thr Gly  
 370 375 380  
 Gln Leu Lys Leu Gln Asn Gly His Val Leu Gln Leu Glu Ser Ala Ser  
 385 390 395 400  
 Asp Lys Ala His Tyr Ile Leu Ser Lys Asp Gly Asn Arg Asn Asn Trp  
 405 410 415  
 Tyr Ile Gly Arg Gly Ser Asp Asn Asn Asn Asp Cys Thr Phe His Ser  
 420 425 430  
 Tyr Val His Gly Thr Thr Leu Thr Leu Lys Gln Asp Tyr Ala Val Val  
 435 440 445  
 Asn Lys His Phe His Val Gly Gln Ala Val Val Ala Thr Asp Gly Asn  
 450 455 460  
 Ile Gln Gly Thr Lys Trp Gly Gly Lys Trp Leu Asp Ala Tyr Leu Arg  
 465 470 475 480  
 Asp Ser Phe Val Ala Lys Ser Lys Ala Trp Thr Gln Val Trp Ser Gly  
 485 490 495  
 Ser Ala Gly Gly Gly Val Ser Val Thr Val Ser Gln Asp Leu Arg Phe  
 500 505 510

seq id.ST25

Arg Asn Ile Trp Ile Lys Cys Ala Asn Asn Ser Trp Asn Phe Phe Arg  
515 520 525

Thr Gly Pro Asp Gly Ile Tyr Phe Ile Ala Ser Asp Gly Gly Trp Leu  
530 535 540

Arg Phe Gln Ile His Ser Asn Gly Leu Gly Phe Lys Asn Ile Ala Asp  
545 550 555 560

Ser Arg Ser Val Pro Asn Ala Ile Met Val Glu Asn Glu  
565 570

<210> 5  
<211> 160  
<212> PRT  
<213> Bacteriophage T7

<400> 5

Met Gly Ser Ser His His His His His His Ser Ser Gly Leu Val Pro  
1 5 10 15

Arg Gly Ser His Pro Phe Gly Gln Leu Lys Thr Met Asn Gln Asn Ser  
20 25 30

Trp Gln Ala Arg Asn Glu Ala Leu Gln Phe Arg Asn Glu Ala Glu Thr  
35 40 45

Phe Arg Asn Gln Ala Glu Gly Phe Lys Asn Glu Ser Ser Thr Asn Ala  
50 55 60

Thr Asn Thr Lys Gln Trp Arg Asp Glu Thr Lys Gly Phe Arg Asp Glu  
65 70 75 80

Ala Lys Arg Phe Lys Asn Thr Ala Gly Gln Tyr Ala Thr Ser Ala Gly  
85 90 95

Asn Ser Ala Ser Ala Ala His Gln Ser Glu Val Asn Ala Glu Asn Ser  
100 105 110

Ala Thr Ala Ser Ala Asn Ser Ala His Leu Ala Glu Gln Gln Ala Asp  
115 120 125

Arg Ala Glu Arg Glu Ala Asp Lys Leu Glu Asn Tyr Asn Gly Leu Ala  
130 135 140

Gly Ala Ile Asp Lys Val Asp Gly Thr Asn Val Tyr Trp Lys Gly Asn  
145 150 155 160

seq id.ST25

<210> 6  
 <211> 309  
 <212> PRT  
 <213> Bacteriophage T7

<400> 6

Met Gly Ser Ser His His His His His Ser Ser Gly Leu Val Pro  
 1 5 10 15

Arg Gly Ser His Met Ala Asn Val Ile Lys Thr Val Leu Thr Tyr Gln  
 20 25 30

Leu Asp Gly Ser Asn Arg Asp Phe Asn Ile Pro Phe Glu Tyr Leu Ala  
 35 40 45

Arg Lys Phe Val Val Val Thr Leu Ile Gly Val Asp Arg Lys Val Leu  
 50 55 60

Thr Ile Asn Thr Asp Tyr Arg Phe Ala Thr Arg Thr Thr Ile Ser Leu  
 65 70 75 80

Thr Lys Ala Trp Gly Pro Ala Asp Gly Tyr Thr Thr Ile Glu Leu Arg  
 85 90 95

Arg Val Thr Ser Thr Thr Asp Arg Leu Val Asp Phe Thr Asp Gly Ser  
 100 105 110

Ile Leu Arg Ala Tyr Asp Leu Asn Val Ala Gln Ile Gln Thr Met His  
 115 120 125

Val Ala Glu Glu Ala Arg Asp Leu Thr Thr Asp Thr Ile Gly Val Asn  
 130 135 140

Asn Asp Gly His Leu Asp Ala Arg Gly Arg Arg Ile Val Asn Leu Ala  
 145 150 155 160

Asn Ala Val Asp Asp Arg Asp Ala Val Pro Phe Gly Gln Leu Lys Thr  
 165 170 175

Met Asn Gln Asn Ser Trp Gln Ala Arg Asn Glu Ala Leu Gln Phe Arg  
 180 185 190

Asn Glu Ala Glu Thr Phe Arg Asn Gln Ala Glu Gly Phe Lys Asn Glu  
 195 200 205

Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp Glu Thr Lys  
 210 215 220

seq id.ST25

Gly Phe Arg Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala Gly Gln Tyr  
225 230 235 240

Ala Thr Ser Ala Gly Asn Ser Ala Ser Ala Ala His Gln Ser Glu Val  
245 250 255

Asn Ala Glu Asn Ser Ala Thr Ala Ser Ala Asn Ser Ala His Leu Ala  
260 265 270

Glu Gln Gln Ala Asp Arg Ala Glu Arg Glu Ala Asp Lys Leu Glu Asn  
275 280 285

Tyr Asn Gly Leu Ala Gly Ala Ile Asp Lys Val Asp Gly Thr Asn Val  
290 295 300

Tyr Trp Lys Gly Asn  
305

<210> 7  
<211> 424  
<212> PRT  
<213> Bacteriophage T7

<400> 7

Met Gly Ser Ser His His His His His His Ser Ser Gly Leu Val Pro  
1 5 10 15

Arg Gly Ser His Pro Phe Gly Gln Leu Lys Thr Met Asn Gln Asn Ser  
20 25 30

Trp Gln Ala Arg Asn Glu Ala Leu Gln Phe Arg Asn Glu Ala Glu Thr  
35 40 45

Phe Arg Asn Gln Ala Glu Gly Phe Lys Asn Glu Ser Ser Thr Asn Ala  
50 55 60

Thr Asn Thr Lys Gln Trp Arg Asp Glu Thr Lys Gly Phe Arg Asp Glu  
65 70 75 80

Ala Lys Arg Phe Lys Asn Thr Ala Gly Gln Tyr Ala Thr Ser Ala Gly  
85 90 95

Asn Ser Ala Ser Ala Ala His Gln Ser Glu Val Asn Ala Glu Asn Ser  
100 105 110

Ala Thr Ala Ser Ala Asn Ser Ala His Leu Ala Glu Gln Gln Ala Asp  
115 120 125

seq id.ST25

Arg Ala Glu Arg Glu Ala Asp Lys Leu Glu Asn Tyr Asn Gly Leu Ala  
 130 135 140  
 Gly Ala Ile Asp Lys Val Asp Gly Thr Asn Val Tyr Trp Lys Gly Asn  
 145 150 155 160  
 Ile His Ala Asn Gly Arg Leu Tyr Met Thr Thr Asn Gly Phe Asp Cys  
 165 170 175  
 Gly Gln Tyr Gln Gln Phe Phe Gly Gly Val Thr Asn Arg Tyr Ser Val  
 180 185 190  
 Met Glu Trp Gly Asp Glu Asn Gly Trp Leu Met Tyr Val Gln Arg Arg  
 195 200 205  
 Glu Trp Thr Thr Ala Ile Gly Gly Asn Ile Gln Leu Val Val Asn Gly  
 210 215 220  
 Gln Ile Ile Thr Gln Gly Gly Ala Met Thr Gly Gln Leu Lys Leu Gln  
 225 230 235 240  
 Asn Gly His Val Leu Gln Leu Glu Ser Ala Ser Asp Lys Ala His Tyr  
 245 250 255  
 Ile Leu Ser Lys Asp Gly Asn Arg Asn Asn Trp Tyr Ile Gly Arg Gly  
 260 265 270  
 Ser Asp Asn Asn Asn Asp Cys Thr Phe His Ser Tyr Val His Gly Thr  
 275 280 285  
 Thr Leu Thr Leu Lys Gln Asp Tyr Ala Val Val Asn Lys His Phe His  
 290 295 300  
 Val Gly Gln Ala Val Val Ala Thr Asp Gly Asn Ile Gln Gly Thr Lys  
 305 310 315 320  
 Trp Gly Gly Lys Trp Leu Asp Ala Tyr Leu Arg Asp Ser Phe Val Ala  
 325 330 335  
 Lys Ser Lys Ala Trp Thr Gln Val Trp Ser Gly Ser Ala Gly Gly Gly  
 340 345 350  
 Val Ser Val Thr Val Ser Gln Asp Leu Arg Phe Arg Asn Ile Trp Ile  
 355 360 365  
 Lys Cys Ala Asn Asn Ser Trp Asn Phe Phe Arg Thr Gly Pro Asp Gly  
 Page 7

370

375

seq id.ST25  
380

Ile Tyr Phe Ile Ala Ser Asp Gly Gly Trp Leu Arg Phe Gln Ile His  
385 390 395 400

Ser Asn Gly Leu Gly Phe Lys Asn Ile Ala Asp Ser Arg Ser Val Pro  
405 410 415

Asn Ala Ile Met Val Glu Asn Glu  
420

<210> 8  
<211> 31  
<212> DNA  
<213> Aequorea victoria

<400> 8  
cggtcgccca tatggtgagc aagggcgagg a 31

<210> 9  
<211> 36  
<212> DNA  
<213> Aequorea victoria

<400> 9  
gattatgatc atatgtctag atccggtgga tcctac 36

<210> 10  
<211> 43  
<212> DNA  
<213> Homo sapiens

<400> 10  
gctgctctca tatgtgtgat ctgcctcaaa cccacagcct ggg 43

<210> 11  
<211> 52  
<212> DNA  
<213> Homo sapiens

<400> 11  
tgaaccagca tatgttcctt acttcttaaa ctttcttgca agtttggtga ca 52

<210> 12  
<211> 36  
<212> DNA  
<213> Bacteriophage T7

<400> 12  
ggaattccat atgtgtgatg ctgttccggt tggtca 36

<210> 13  
<211> 32  
<212> DNA



&lt;213&gt; Bacteriophage T7

&lt;400&gt; 13

cgcggatcct tagtattgac cagccgtatt ct

32

&lt;210&gt; 14

&lt;211&gt; 33

&lt;212&gt; PRT

&lt;213&gt; Bacteriophage T7

&lt;400&gt; 14

Lys	Asn	Glu	Ser	Ser	Thr	Asn	Ala	Thr	Asn	Thr	Lys	Gln	Trp	Arg	Asp
1				5					10					15	

Glu	Thr	Lys	Gly	Phe	Arg	Asp	Glu	Ala	Arg	Arg	Phe	Lys	Asn	Thr	Ala
			20					25					30		

Gly

&lt;210&gt; 15

&lt;211&gt; 33

&lt;212&gt; PRT

&lt;213&gt; Bacteriophage T7

&lt;400&gt; 15

Lys	Asn	Glu	Ser	Ser	Thr	Asn	Ala	Thr	Asn	Thr	Lys	Gln	Trp	Lys	Asp
1				5					10					15	

Glu	Thr	Lys	Gly	Phe	Arg	Asp	Glu	Ala	Lys	Arg	Phe	Lys	Asn	Thr	Ala
			20					25					30		

Gly

&lt;210&gt; 16

&lt;211&gt; 40

&lt;212&gt; PRT

&lt;213&gt; Bacteriophage T7

&lt;400&gt; 16

Lys	Asn	Glu	Ser	Ser	Thr	Asn	Ala	Thr	Asn	Thr	Lys	Gln	Trp	Arg	Asp
1				5					10					15	

Glu	Thr	Lys	Gly	Phe	Arg	Asp	Glu	Ala	Lys	Arg	Phe	Lys	Asn	Thr	Ala
			20					25					30		

Gly	Gln	Tyr	Ala	Thr	Ser	Ala	Gly
		35					40

seq id.ST25

<210> 17  
 <211> 33  
 <212> PRT  
 <213> Bacteriophage T7

<400> 17

Lys Asn Glu Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp  
 1 5 10 15

Glu Thr Lys Gly Phe Arg Asp Glu Ala Lys Arg Phe Lys Asp Glu Ala  
 20 25 30

Gly

<210> 18  
 <211> 28  
 <212> PRT  
 <213> Bacteriophage T7

<400> 18

Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp Glu Thr Lys Gly Phe  
 1 5 10 15

Arg Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala Gly  
 20 25

<210> 19  
 <211> 29  
 <212> PRT  
 <213> Bacteriophage T7

<400> 19

Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp Glu Thr Lys Gly  
 1 5 10 15

Phe Arg Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala Gly  
 20 25

<210> 20  
 <211> 30  
 <212> PRT  
 <213> Bacteriophage T7

<400> 20

Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp Glu Thr Lys  
 1 5 10 15

Gly Phe Arg Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala Gly  
 20 25 30

seq id.ST25

<210> 21  
 <211> 31  
 <212> PRT  
 <213> Bacteriophage T7

<400> 21

Glu Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp Glu Thr  
 1 5 10 15

Lys Gly Phe Arg Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala Gly  
 20 25 30

<210> 22  
 <211> 33  
 <212> PRT  
 <213> Bacteriophage T7

<400> 22

Lys Asn Glu Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp  
 1 5 10 15

Glu Thr Lys Gly Phe Arg Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala  
 20 25 30

Gly

<210> 23  
 <211> 33  
 <212> PRT  
 <213> Bacteriophage T7

<400> 23

Lys Asn Glu Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp  
 1 5 10 15

Glu Thr Lys Gly Phe Arg Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala  
 20 25 30

Gly

<210> 24  
 <211> 28  
 <212> PRT  
 <213> Bacteriophage T7

<400> 24

Lys Asn Glu Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp  
 1 5 10 15

seq id.ST25

Glu Thr Lys Gly Phe Arg Asp Glu Ala Lys Arg Phe  
20 25

<210> 25  
<211> 33  
<212> PRT  
<213> Bacteriophage T7

<400> 25

Lys Asn Glu Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp  
1 5 10 15

Glu Thr Lys Gly Phe Arg Asp Glu Ala Glu Arg Phe Lys Asn Thr Ala  
20 25 30

Gly

<210> 26  
<211> 33  
<212> PRT  
<213> Bacteriophage T7

<400> 26

Arg Asn Glu Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp  
1 5 10 15

Glu Thr Lys Gly Phe Arg Asp Glu Ala Arg Arg Phe Arg Asn Thr Ala  
20 25 30

Gly

<210> 27  
<211> 33  
<212> PRT  
<213> Bacteriophage T7

<400> 27

Lys Asn Glu Ser Ser Thr Asn Ala Thr Asn Thr Arg Gln Trp Arg Asp  
1 5 10 15

Glu Thr Arg Gly Phe Arg Asp Glu Ala Arg Arg Phe Lys Asn Thr Ala  
20 25 30

Gly

seq id.ST25

<210> 28  
 <211> 25  
 <212> PRT  
 <213> Bacteriophage T7

<400> 28

Lys Asn Glu Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp  
 1 5 10 15

Glu Thr Lys Gly Phe Arg Asp Glu Ala  
 20 25

<210> 29  
 <211> 25  
 <212> PRT  
 <213> Bacteriophage T7

<400> 29

Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp Glu Thr Lys  
 1 5 10 15

Gly Phe Arg Asp Glu Ala Lys Arg Phe  
 20 25

<210> 30  
 <211> 28  
 <212> PRT  
 <213> Bacteriophage T7

<400> 30

Lys Asn Glu Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp  
 1 5 10 15

Glu Thr Lys Gly Phe Arg Asp Glu Ala Lys Arg Phe  
 20 25

<210> 31  
 <211> 15  
 <212> PRT  
 <213> Bacteriophage T7

<400> 31

Lys Gly Phe Arg Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala Gly  
 1 5 10 15

<210> 32  
 <211> 22  
 <212> PRT  
 <213> Bacteriophage T7

<400> 32

seq id.ST25

Lys Gln Trp Arg Asp Glu Thr Lys Gly Phe Arg Asp Glu Ala Lys Arg  
 1 5 10 15

Phe Lys Asn Thr Ala Gly  
 20

<210> 33  
 <211> 29  
 <212> PRT  
 <213> Bacteriophage T7

<400> 33

Leu Lys Thr Met Asn Gln Asn Ser Trp Gln Ala Arg Asn Glu Ala Leu  
 1 5 10 15

Gln Phe Arg Asn Glu Ala Glu Thr Gly Arg Asn Gln Ala  
 20 25

<210> 34  
 <211> 33  
 <212> PRT  
 <213> Bacteriophage T7

<400> 34

Ala Thr Asn Thr Lys Gln Trp Arg Asp Glu Thr Lys Gly Phe Arg Asp  
 1 5 10 15

Glu Ala Lys Arg Phe Lys Asn Thr Ala Gly Gln Tyr Ala Thr Ser Ala  
 20 25 30

Gly

<210> 35  
 <211> 27  
 <212> PRT  
 <213> Bacteriophage T7

<400> 35

Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp Glu Thr Lys Gly Phe Arg  
 1 5 10 15

Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala Gly  
 20 25

<210> 36  
 <211> 33  
 <212> PRT  
 <213> Bacteriophage T7

<400> 36

seq id.ST25

Lys Asn Glu Ser Ser Thr Asn Ala Thr Asn Thr Lys Gln Trp Arg Asp  
 1 5 10 15

Glu Thr Lys Gly Phe Arg Asp Glu Ala Lys Arg Phe Lys Asn Thr Ala  
 20 25 30

Gly